Stem cells treat life-threatening skin condition

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This week researchers at the University of Minnesota published a paper showing that stem cells from the bone marrow can help kids with a blistering skin condition called epidermolysis bullosa. The disease is horrible. Lacking a protein to anchor skin in place, the children's blister at the slightest touch -- on their skin, in their throat, inside their eyelids, and anywhere else skin forms.

The group gave the kids a bone marrow transplant, replacing their own blood system with cells that make the form of collagen lacking in kids with the disease. It worked. In a press release, John Wagner, M.D., director of pediatric blood and marrow transplantation and clinical director of the Stem Cell Institute, said:

> "To understand this achievement, you have to understand how horrible this disease actually is. From the moment of birth, these children develop blisters from the slightest trauma which eventually scar. They live lives of chronic pain, preventing any chance for a normal life. My hope is to do something that might change the natural history of this disease and enhance the quality of life of these kids."

A Canadian CBC news story quotes Pediatric dermatologist Dr. Elena Pope, medical director of the EB clinic at Toronto's Hospital for Sick Children, as saying:

> "It's extremely, extremely exciting for us who are working in this area to actually see some steps forward."

CIRM funds a disease team headed by Alfred Lane at Stanford Univerversity, who is also working toward a stem cell-based therapy for the disease. His team is creating reprogrammed iPS cells from the children's skin, inserting a good copy of the mutated gene, and transplanting the resulting skin cells back onto the children.

CIRM's epidermolysis bullosa disease team: Anthony Oro, Gerhard Bauer, Alfred Lane, Marius Wernig

Whichever approach is successful long-term, it's nice to see progress being made for this truly horrible disease.

A.A.

Tags: Disease Team, Lane, Stanford University, epidermolysis bullosa